

Clinical Trials for Osteoarthritis, Multiple Myeloma and Making Bone Marrow Transplants Safer Get Go-ahead from Stem Cell Agency Board.

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October 26, 2017 Oakland, CA An estimated 30 million Americans suffer from osteoarthritis. It's a painful, often debilitating, condition caused when the cartilage or cushion between our joints breaks down, leaving bone to rub on bone. Osteoarthritis is a leading cause of hospitalization and results in 600,000 Americans undergoing knee replacement surgery. Now the California Institute for Regenerative Medicine (CIRM), the state's Stem Cell Agency, hopes to change that by investing in a less invasive stem cell-based option to treat osteoarthritis.

Researchers at the California Institute for Biomedical Research (CALIBR) have been awarded \$8.447 million to test KA34, a drug that, in preclinical tests, recruits stem cells to create new cartilage in areas damaged by osteoarthritis. CIRM funded the research that developed this technology and now this Phase 1 trial will test this stem cell-directed treatment in people with osteoarthritis of the knee, hopefully slowing down or even halting the progression of the disease.

The CIRM Board also awarded Poseida Therapeutics almost \$20 million to assess the safety of a gene modified cell therapy to treat multiple myeloma, the abnormal growth of malignant plasma cells of the immune system.

"Multiple myeloma disproportionately affects people over the age of 65 and African Americans, and it leads to progressive bone destruction, severe anemia, infectious complications and kidney and heart damage from abnormal proteins produced by the malignant plasma cells. Less than half of patients with multiple myeloma live beyond 5 years," says Maria Millan, M.D., President and CEO of CIRM. "Poseida's technology is seeking to destroy these cancerous myeloma cells with an immunotherapy approach that uses the patient's own engineered immune system T cells to seek and destroy the myeloma cells."

The third clinical program approved by the CIRM Board also involves the use of T cells, but instead of targeting cancer this approach will help fight active viral infections. Viruses such cytomegalovirus, Epstein-Barr virus, and adenovirus commonly infect and lead to fatal complications in patients with weakened immune systems resulting from chemotherapy, bone marrow or cord blood transplant, and other forms of inherited or acquired disorders.

A team at Children's Hospital of Los Angeles is being awarded \$4.8 million for a Phase 1 clinical trial to test the feasibility of providing these immune suppressed patients with engineered T-cells to fight these viruses. Donated virus-specific T-cells will be matched to the patient's immune system to help boost their ability to fight off these viruses and to provide longer-term anti-viral protection.

Approval of these three projects means that CIRM has now funded 43 clinical trials overall, 16 in 2017 alone. You can find out more information about all these trials on CIRM's Clinical Dashboard.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information, go to www.cirm.ca.gov